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Award Number: DAMD17-02-1-0654

TITLE: Health-Related Quality of Life for Pediatric NF1 Patients

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REPORT DATE: August 2004

TYPE OF REPORT: Annual

PREPARED FOR: U.S. Army Medical Research and Materiel Command
Fort Detrick, Maryland 21702-5012

DISTRIBUTION STATEMENT: Approved for Public Release;
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20050603 107

REPORT DOCUMENTATION PAGE

*Form Approved
OMB No. 074-0188*

| | | |
|--|---|---|
| Public reporting burden for this collection of information is estimated to average 1 hour per response, including the time for reviewing instructions, searching existing data sources, gathering and maintaining the data needed, and completing and reviewing this collection of information. Send comments regarding this burden estimate or any other aspect of this collection of information, including suggestions for reducing this burden to Washington Headquarters Services, Directorate for Information Operations and Reports, 1215 Jefferson Davis Highway, Suite 1204, Arlington, VA 22202-4302, and to the Office of Management and Budget, Paperwork Reduction Project (0704-0188), Washington, DC 20503. | | |
| 1. AGENCY USE ONLY (Leave blank) | 2. REPORT DATE August 2004 | 3. REPORT TYPE AND DATES COVERED Annual (1 Aug 03 – 30 Jul 04) |
| 4. TITLE AND SUBTITLE Health-Related Quality of Life for Pediatric NF1 Patients | | 5. FUNDING NUMBERS DAMD17-02-1-0654 |
| 6. AUTHOR(S) Andrew S. Bradlyn, Ph.D. Carole V. Harris, Ph.D. | | |
| 7. PERFORMING ORGANIZATION NAME(S) AND ADDRESS(ES) West Virginia University Research Corporation Morgantown, West Virginia 26506-6845 E-Mail: abradlyn@hsc.wvu.edu | | 8. PERFORMING ORGANIZATION REPORT NUMBER |
| 9. SPONSORING / MONITORING AGENCY NAME(S) AND ADDRESS(ES) U.S. Army Medical Research and Materiel Command Fort Detrick, Maryland 21702-5012 | | 10. SPONSORING / MONITORING AGENCY REPORT NUMBER |
| 11. SUPPLEMENTARY NOTES | | |
| 12a. DISTRIBUTION / AVAILABILITY STATEMENT Approved for Public Release; Distribution Unlimited | | 12b. DISTRIBUTION CODE |
| 13. ABSTRACT (Maximum 200 Words) The objective of this project is to develop an NF1-specific health-related quality of life (HRQL) instrument for use with pediatric patients. Semi-structured interviews with children with NF1, their parents, and teachers have been used to derive important domains and item content, and have been used to develop the first-generation instrument. This instrument will undergo an initial examination of its psychometric properties and the content will be revised accordingly. Following this initial field trial, the second generation instrument will be tested administered in a battery of measures to further assess its reliability and validity as well as its applicability in a clinical trials setting. At the conclusion of this second year, we have completed all interviews, transcribed all interviews, and reviewed them for content. This content review was then used to develop the first-generation instrument. We have obtained local (WVU) Institutional Review Board approval for testing this instrument, after having made modifications to the protocol as requested by the Department of Defense review. At this point, the amended protocol has been approved by the local IRB and we await final Department of Defense approval to recruit participants. | | |
| 14. SUBJECT TERMS Quality of Life | | 15. NUMBER OF PAGES 36 |
| | | 16. PRICE CODE |
| 17. SECURITY CLASSIFICATION OF REPORT Unclassified | 18. SECURITY CLASSIFICATION OF THIS PAGE Unclassified | 19. SECURITY CLASSIFICATION OF ABSTRACT Unclassified |
| | | 20. LIMITATION OF ABSTRACT Unlimited |

NSN 7540-01-280-5500

Standard Form 298 (Rev. 2-89)
Prescribed by ANSI Std. Z39-18
298-102

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Andrew S. Bradlyn, Ph.D., Principal Investigator
Annual Report Year 2

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1. INTRODUCTION

The objective of this project is to develop an NF1-specific health-related quality of life (HRQL) instrument for pediatric patients. Semi-structured interviews with children with NF-1, their parents, and teachers have yielded relevant domains and content that have been used to develop the first-generation instrument. This first-generation instrument will be field tested for psychometric properties and revised. Finally, we will administer the revised instrument to a group of patients and their parents and test its validity and sensitivity to change in clinical status. It is our hypothesis that the addition of this disease-specific measure will significantly improve the descriptiveness and quantification of the impact of NF-1 and its treatment on children's lives.

2. BODY

The original Statement of Work identified the following tasks in the first two years:

- Complete review of the literature to gather previously-identified HRQL-related topics (completed in Year 1);
- Develop standardized interviews for participants;
- Pilot interviews and modify as needed;
- Conduct interviews;
- Transcribe interviews and review for functioning areas impacted by NF-1
- Recruit NF1 participants and parents
- Mail out questionnaires for completion and return
- Enter and analyze data
- Pilot NF1-instrument specific format, instructions, and interface
- Produce final NF1-specific instrument for studies in subsequent Tasks

Additionally, although not stated in the original SoW, we were required to gain approval from both the Army/Department of Defense Human Subjects Review Board and our local Institutional Review Board prior to recruiting participants. It was necessary to submit separate applications for each of the two projects (interviews and first-generation measure).

Status of Work. Each of the above tasks is listed again, with current status described.

Task 1: Identify domains and items to reflect important aspects of HRQL.

- *Complete review of the literature to gather previously-identified HRQL-related topics (completed in Year 1).* As noted in our prior Annual Report, this was completed in Year 1.
- *Develop standardized interviews for participants.* This task was completed in Year 1, however the interview guide for the child participants

was modified to be more "child friendly" as suggested in the Year 1 review.

- *Pilot interviews and modify as needed.* Completed in Year 1.
- *Conduct interviews.* We continued to recruit participants for the interview portion of the project throughout much of Year 2, and have now completed this task. As noted in our prior report, we recruited participants through web sites (e.g., Neurofibromatosis Foundation; Neurofibromatosis, Inc), web bulletin boards, mailings and contacts with NF clinics and state associations, and presentations. The following table provides descriptive information about the participants:

| Variable | Description |
|--------------------------------|--|
| Parent Participants | N = 25 |
| Gender | 3 male; 22 female |
| Age of Child | M = 13.1 years |
| Severity of Child (Riccardi) | Range = 1 – 4 Mode = 1 |
| Child Participants (ages 8-17) | N = 20 |
| Age | M = 12.34 <u>sd</u> = 2.6 Range = 8 – 16 |
| Gender | 8 male 12 female |
| Severity (Riccardi) | Range = 1 – 4 Mode = 1 |
| Teacher Participants | N = 2 |

- *Transcribe interviews and review for functioning areas impacted by NF-1.* This task has been completed; interviews were continued to the point of redundancy (i.e., no new information elicited). All interviews were transcribed and reviewed and items were written to reflect the domains and issues that were derived from the interviews and from the existing literature.
- Task 2: Administer preliminary items to a sample of NF1 patients and parents and use item analysis to construct preliminary HRQL instrument. We are currently awaiting final DoD IRB approval to begin recruiting participants for this task. We have experienced delays in recruiting for both Task 1 and Task 2 due to IRB timelines. More specifically, we have had to submit separate applications for each of the tasks (interviews and preliminary instrument testing) and the application for Task 2 could not be submitted until the interviews for Task 1 had been completed, transcribed and reviewed, as the preliminary instrument could not be written (and reviewed by the IRB) until that work was completed. After securing

approval from the local (WVU) IRB, the application to the Department of Defense was submitted; following the DoD review and comments, we made the suggested revisions and then submitted amendments to the WVU IRB. After we received approval for this amended application, we then forwarded the materials to the DoD for re-review and approval. This process has resulted in delays in recruiting, however we have a list of potential participants who had expressed interest in receiving more information and we have mailings and web postings ready to distribute pending final DoD approval. Importantly, the preliminary instrument has been drafted and reviewed and is ready for testing.

Following approval, we will be recruiting participants to complete this first-generation instrument. Children between the ages of 8 and 17 years, and parents of youth with NF-1 will be recruited to the study. Participants will be recruited from web postings, mailings, and NF clinics. Responses to the questionnaire will be subjected to standard item analysis procedures, primarily examining the distribution of item and subscale responses. Items which evidence overly restricted ranges will be reviewed and considered for elimination if not judged to be key. Items which are noted to be confusing to participants will either be re-written and clarified or dropped. This remaining group of items will constitute the final questionnaire to be used in Task 3.

In light of the delays we have experienced, we anticipate that it will be necessary to request an extension on the project (no cost) to complete Task 3.

Results and Discussion of Research Activities. The primary findings from the current work relate to the specific themes and issues identified by participants. Children, youth and parents responded to a broad range of interview questions, designed to elicit information regarding physical, social, and emotional functioning (the three core areas of health as defined by the World Health Organization). Interviews with teachers covered a similar breadth of topics, but the questions emphasized the child's functioning in the school setting. The concerns identified in the interviews are reflected in the item content of the questionnaires to be used in Phase 2 (provided as attachments).

The following themes and specific issues were identified from interviews with children, youth and families (checkmarks indicate that at least one participant in that specific group reported difficulties):

| Theme | Parents | Children (8-12) | Youth (13-17) | Teachers | Comments Examples |
|-------------------|---------|-----------------|---------------|----------|---|
| Understanding NF1 | ✓ | ✓ | | | Frustration with having NF1, difficulty understanding the disease |

| | | | | | |
|--|---|---|---|---|--|
| Problems with sleep | ✓ | ✓ | ✓ | | Difficulties falling asleep, maintaining sleep, or early morning awakening |
| Problems with appetite | ✓ | ✓ | | | |
| Problems with sensation | ✓ | ✓ | ✓ | ✓ | Sight, hearing, touch, |
| Pain | ✓ | ✓ | ✓ | | Related to tumors, surgeries, nerve involvement |
| Specific physical symptoms | ✓ | ✓ | ✓ | ✓ | NF1-specific symptoms; other miscellaneous |
| Specific psychological or behavioral symptoms | ✓ | ✓ | ✓ | ✓ | Symptoms related to Attention Deficit Hyperactivity Disorder, Pervasive Developmental Disorders, Mood Disorders, Anxiety Disorders |
| Specific learning/cognitive problems and/or classroom difficulties | ✓ | ✓ | ✓ | ✓ | Reading, writing, attendance, learning disability, classroom modifications |
| Social concerns | ✓ | ✓ | ✓ | ✓ | Difficulties with family members and/or friends; preferring solitary activities; teasing |
| Fine and/or gross motor coordination | ✓ | ✓ | ✓ | ✓ | Handwriting, running, walking, clumsiness |
| Concerns about physical appearance | ✓ | ✓ | ✓ | | Café-au-lait spots, stature, over/underweight, head size |
| Difficulties with speech and language | ✓ | ✓ | ✓ | ✓ | Articulation problems |

In terms of physical impairments, several issues were commonly identified. In particular, several participants reported significant concerns regarding ongoing, chronic pain and acute recurrent pains (e.g., headache). Motor problems, such as poor fine and gross motor coordination were commonly noted by parents, children and teachers; these were evidenced by clumsiness and handwriting problems, for example. A number of children and parents reported difficulties with sleep (either initiating or maintaining sleep), and sensory difficulties (auditory or visual) were frequently endorsed. As noted below, although over- or hyperactivity was reported (often associated with the diagnosis of Attention Deficit Hyperactivity Disorder), several participants noted easy fatigability, to the point of being an impediment to participation in day-to-day activities. Finally, participants

noted a wide range of stature and weight concerns, ranging including short-stature and both over- and under-weight status, which was often attributed to NF1.

Psychological impairments reported included a number of diagnosed psychiatric disorders, including Attention Deficit Hyperactivity Disorder, Mood Disorders (e.g., Major Depressive Disorder, Bipolar Affective Disorder), Pervasive Developmental Disorders, and Anxiety Disorders (e.g., Panic Disorder). Participants were often unclear as to whether these were related to NF1; however, they did note impairments in school and social relations, in particular that were related to these disorders.

Social impairments were reported by participants, as well. These often included situations in which individuals were teased by peers regarding their physical appearance. Additionally, a number of participants noted a preference for solitary activities, as compared to group socialization. It is important to note however, that a substantial number of children and youth described themselves as very outgoing and engaging socially, and that they perceived their peers and family as being supportive.

In their consensus statement, the NF1 Cognitive Disorders Task Force (North et al., 1997a) concluded that there was a high incidence of learning disabilities (30-65%) in individuals with NF, as compared to the general population. Our data are consistent with this finding as well, as a number of participants (children, youth, parents and teachers) noted a range of cognitive difficulties that were often associated with impaired school performance. We frequently noted children and youth who received special education services as well, whether it was in the form of full-time self-contained classes or domain-specific assistance (e.g., reading or mathematics support).

In terms of behavioral difficulties, the themes and issues identified by our participants were generally consistent with the pattern that has emerged in the scientific literature. For example, Wadsby et al (1989) reported that children with NF were more likely than their siblings to have difficulties with sleeping, disruptive behaviors, and distractibility. Although not conclusive, there are data to suggest a high incidence of ADHD in children with NF (cf., Moore et al., 1996; North et al., 1997b) and participants in our interviews frequently reported either a formal diagnosis of ADHD or symptoms consistent with this diagnosis. Reporting on an investigation comparing NF1 individuals with non-affected siblings and parents, Koth et al., (2000) concluded that ADHD may occur as a component of NF1.

Although the findings were limited by a relatively small sample size, Spaepen, Borghgraef, and Fryns (1992) found that approximately 50% of their sample had parent-reported behavior problems that were at a level similar to children referred

for psychiatric treatment. Problems reported in this sample included social withdrawal, aggressiveness, anxiety, and somatic complaints.

The physical symptoms reported by our participants were generally consistent with what might be predicted for individuals with NF1 and a host of co-morbid conditions. Headaches, short stature, macrocephaly and seizures are frequently noted manifestations of the disease (cf., Goldberg et al., 1996), and were reported as concerns by parents, youth and children.

Overall, the information gathered from the interviews was generally consistent with published data regarding the effects of NF1 on individuals. The interviews do provide a context for understanding the impact of the disease however, and in particular highlight the spectrum of effects (from minimal to significant impairment) that may be experienced with this disease. Anecdotally, we also noted that parents and their children often identified similar issues, which is an important factor to be considered in developing child-report and parent-proxy report measures of functioning.

3. KEY RESEARCH ACCOMPLISHMENTS

There are two primary accomplishments to report: (a) information derived from parent and child interviews regarding the impact of NF1 on day-to-day functioning, and (b) development of the preliminary instrument to assess HRQL for both child (8-17 years) and parent-proxy report.

4. REPORTABLE OUTCOMES AND CONCLUSIONS

The child and parent interviews are being used to prepare a manuscript regarding the qualitative analyses of the information that was obtained. In addition to the expected range of physical problems that were encountered, there was a great deal of information regarding the psychosocial morbidity associated with NF1. For example, children (and parents) reported difficult peer relations and social problems, but also provided many illustrations of coping and resilience. Additionally, a number of children had been diagnosed with psychiatric disorders, often externalizing disorders such as Attention Deficit/Hyperactivity Disorder, but also internalizing problems such as mood disorders and anxiety disorders. Managing the combined impact of these co-morbid psychiatric and general medical disorders appears to be significant and challenging for many of these families. Our preliminary instrument includes sections on peer and social relations, but also on basic, broad psychopathology in order that these problem behaviors can be incorporated into any quantification of health-related quality of life.

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6. APPENDICES

We have attached the preliminary HRQL instrument for child and parent-proxy report.

NF-1 Health-Related Quality of Life

Version 1.0

PARENT REPORT

DIRECTIONS:

Children with NF-1 sometimes have special problems. We are interested in how much of a problem these might have been for your child in the PAST WEEK. There are no right or wrong answers! Just circle the number that answers the question. If the question doesn't make sense to you, just circle "I don't understand." Thanks!

GENERAL HEALTH

In general, I would say that my child's health is:

| | | | | | |
|-----------|---|---|---|----------------|-----------------------|
| 1 Poor | 2 | 3 | 4 | 5 Excellent | 1 Don't Understand |
|-----------|---|---|---|----------------|-----------------------|

Compared to this time last year, I would say my child's health is:

| | | | | | |
|-----------------|---|---|---|------------------|-----------------------|
| 1 Much worse | 2 | 3 | 4 | 5 Much better | 1 Don't Understand |
|-----------------|---|---|---|------------------|-----------------------|

My child get sick more easily than other kids:

| | | | | | |
|----------------------|---|---|---|---------------------|-----------------------|
| 1 Yes, definately | 2 | 3 | 4 | 5 No, not at all | 1 Don't Understand |
|----------------------|---|---|---|---------------------|-----------------------|

APPETITE AND SLEEP

In the past week, has your child:

| | Always | 2 | Sometimes | 3 | 4 | 5 | Never | Don't Understand | ? |
|--|--------|---|-----------|---|---|---|-------|------------------|---|
| Had difficulty falling asleep or staying asleep? | 1 | | | | | | | | |
| Had bad dreams or nightmares? | 1 | 2 | 3 | 4 | 5 | 5 | 5 | ? | ? |
| Been less hungry than usual? | 1 | 2 | 3 | 4 | 5 | 5 | 5 | ? | ? |
| Been hungrier than usual? | 1 | 2 | 3 | 4 | 5 | 5 | 5 | ? | ? |

APPETITE AND SLEEP

In the past week, has your child:

| | Always | 2 | Sometimes | 4 | 5 | Never | Don't Understand |
|--|--------|---|-----------|---|---|-------|------------------|
| Had difficulty falling asleep or staying asleep? | 1 | 2 | 3 | 4 | 5 | 5 | ? |
| Had bad dreams or nightmares? | 1 | 2 | 3 | 4 | 5 | 5 | ? |
| Been less hungry than usual? | 1 | 2 | 3 | 4 | 5 | 5 | ? |
| Been hungrier than usual? | 1 | 2 | 3 | 4 | 5 | 5 | ? |

PAIN

In the past week, has your child:

| | Always 1 | 2 | Sometimes 3 | 4 | Never 5 | Don't Understand ? |
|---|----------------|---|---------------------|---|-----------------|-----------------------|
| Hurt a lot? | 1 | 2 | 3 | 4 | 5 | ? |
| Had muscle pains or aches | 1 | 2 | 3 | 4 | 5 | ? |
| Had headaches? | 1 | 2 | 3 | 4 | 5 | ? |
| Had stomachaches? | 1 | 2 | 3 | 4 | 5 | ? |
| Had pain in your bones or joints? | 1 | 2 | 3 | 4 | 5 | ? |
| Had neurofibromas or other areas on his/her body that hurt to touch? | 1 | 2 | 3 | 4 | 5 | ? |
| Compared to other people my child's age, the amount of pain my child has is | Much more 1 | 2 | About the same 3 | 4 | Much less 5 | Don't Understand ? |
| Pain keeps my child from doing the things s/he want to do or needs to do | Very true 1 | 2 | Somewhat 3 | 4 | Not at all 5 | Don't Understand ? |

UNDERSTANDING**In the past week, was your child:****Bothered that s/he didn't know enough about NF-1?**

| | | | | | |
|------------|---|-----------|---|-----------------|-----------------------|
| 1 A lot | 2 | 3 Some | 4 | 5 Not at all | 1 Don't Understand |
|------------|---|-----------|---|-----------------|-----------------------|

Frustrated about having NF-1?

| | | | | | |
|------------|---|-----------|---|-----------------|-----------------------|
| 1 A lot | 2 | 3 Some | 4 | 5 Not at all | 1 Don't Understand |
|------------|---|-----------|---|-----------------|-----------------------|

SENSATION**In the past week, has your child:**

Had difficulty hearing others when they talk?
Check here if s/he wears a hearing aid _____

Had trouble seeing well?
Check here if s/he wears glasses or contacts _____

Had trouble feeling the things s/he touches?

| | Always | 2 | Sometimes | 4 | 5 | Never | Don't Understand |
|---|--------|---|-----------|---|---|-------|------------------|
| Had difficulty hearing others when they talk? Check here if s/he wears a hearing aid _____ | 1 | 2 | 3 | 4 | 5 | Never | Don't Understand |
| Had trouble seeing well? Check here if s/he wears glasses or contacts _____ | 1 | 2 | 3 | 4 | 5 | Never | Don't Understand |
| Had trouble feeling the things s/he touches? | 1 | 2 | 3 | 4 | 5 | Never | Don't Understand |

SYMPTOMS

In the past week, has your child:

| | Always | 2 | Sometimes | 4 | 5 | Never | Don't Understand |
|---|--------|---|-----------|---|---|-------|------------------|
| Felt sick to his/her stomach? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had a headache? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had a stomachache? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had a hard time breathing? | 1 | 2 | 3 | 4 | 5 | | ? |
| Felt dizzy or like s/he might faint? | 1 | 2 | 3 | 4 | 5 | | ? |
| Felt weak? | 1 | 2 | 3 | 4 | 5 | | ? |
| Felt tired or fatigued? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had a hard time swallowing? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had a hard time keeping his/her balance? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had difficulty tying shoes, using scissors, holding a pencil? | 1 | 2 | 3 | 4 | 5 | | ? |
| Been clumsy? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had a hard time riding a bike, running, or catching a ball? | 1 | 2 | 3 | 4 | 5 | | ? |

PSYCHOLOGICAL AND BEHAVIORAL-1

In the past week, has your child:

| | Always | 2 | Sometimes | 4 | Never | 5 | Don't Understand |
|--|--------|---|-----------|---|-------|---|------------------|
| Felt cranky or irritable? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Worried? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Felt anxious? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Gotten easily frustrated? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Gotten in trouble because of his/her behavior at school? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Gotten in trouble because of his/her behavior at home? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Felt afraid or scared? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Felt sad, down, or depressed? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Felt angry? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Worried about what might happen to him/her? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Felt like crying? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Felt lonely? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Felt cheerful? | 1 | 2 | 3 | 4 | 5 | ? | ? |

PSYCHOLOGICAL AND BEHAVIORAL -2

In the past week, has your child:

| | Always | 2 | Sometimes | 3 | 4 | 5 | Never | Don't Understand |
|--|-----------------|---|-----------|---|---|------------------|-------|------------------|
| Felt confident about him/herself? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Enjoyed the things s/he does? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Had fun? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Felt jittery or restless? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Argued? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Wanted to be alone? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Had mood swings? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Not done what his/her parent or teacher asked? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Had anxiety or panic attacks? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Hit or kicked someone? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Heard voices that weren't there? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Compared to other children your child's age, would you say his/her behavior is | Much worse 1 | 2 | OK 3 | 4 | 5 | Much better / | 5 | Don't Understand |

SCHOOL AND COGNITION-1

In the past week, has your child:

| | Always | 2 | Sometimes | 4 | 5 | Never | Don't Understand |
|--|--------|---|-----------|---|---|-------|------------------|
| Missed school because of his/her health? Check here if school was on vacation _____ | 1 | 2 | 3 | 4 | 5 | | |
| Been bothered because you missed school? Check here if s/he hasn't missed any school _____ | 1 | 2 | 3 | 4 | 5 | | |
| Missed school to go to the doctor or hospital? | 1 | 2 | 3 | 4 | 5 | | |
| Had difficulty solving math problems? | 1 | 2 | 3 | 4 | 5 | | |
| Had trouble writing papers or reports? | 1 | 2 | 3 | 4 | 5 | | |
| Had trouble following or understanding directions? | 1 | 2 | 3 | 4 | 5 | | |
| Had difficulty remembering what s/he read? | 1 | 2 | 3 | 4 | 5 | | |
| Had trouble reading? | 1 | 2 | 3 | 4 | 5 | | |
| Forgotten things? | 1 | 2 | 3 | 4 | 5 | | |
| Had trouble keeping up with schoolwork? | 1 | 2 | 3 | 4 | 5 | | |
| Had trouble turning schoolwork in on time? | 1 | 2 | 3 | 4 | 5 | | |

SCHOOL AND COGNITION--2

In the past week, has your child:

| | Always | 2 | Sometimes | 4 | 5 | Never | Don't Understand |
|---|-----------|---|-----------|---|---|----------------|------------------|
| Had difficulty paying attention and concentrating in class? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Had trouble writing neatly? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Had a hard time sitting still in class? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Had trouble organizing work or things? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Had trouble spelling? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| How would you rate your child's ability to do schoolwork? | Poor 1 | 2 | OK 3 | 4 | 5 | Excellent I | Don't Understand |

SOCIAL RELATIONS

In the past week, has your child:

| | Always | 2 | 3 | Sometimes | 4 | 5 | Never | Don't Understand |
|--|--------|---|---|-----------|---|---|-----------|------------------|
| Had trouble getting along with other kids? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Helped others? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Preferred to be alone? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Preferred quiet activities? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Avoided doing things with other kids? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Felt bothered because s/he couldn't do the activities they like? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Felt like other kids didn't want to do things with him/her? | 1 | 2 | 3 | 4 | 5 | 5 | ? | ? |
| Felt like other kids didn't want to be his/her friend? | | | | | | | | |
| How would you rate your family's ability to get along with each other? | Poor | 1 | 2 | OK | 3 | 4 | Excellent | 1 |
| How would you rate your child's ability to get along with other kids? | | 1 | 2 | 3 | 4 | 5 | 5 | 1 |

APPEARANCE

In the past week, has your child:

| | Always | 2 | Sometimes | 4 | Never | Don't Understand |
|---|-----------------|---|---------------|---|----------------|-----------------------|
| Been teased about how they look? | 1 | 2 | 3 | 4 | 5 | ? |
| Felt bad about his/her appearance? | 1 | 2 | 3 | 4 | 5 | ? |
| Worried about their looks? | 1 | 2 | 3 | 4 | 5 | ? |
| Been teased about size? | 1 | 2 | 3 | 4 | 5 | ? |
| Avoided doing things with others because of how s/he looks? | 1 | 2 | 3 | 4 | 5 | ? |
| Done things to hide part of their body because of how it looks? | 1 | 2 | 3 | 4 | 5 | ? |
| Noticed people staring at them? | 1 | 2 | 3 | 4 | 5 | ? |
| Heard strangers make rude comments about them? | 1 | 2 | 3 | 4 | 5 | ? |
| Worried about other people teasing them? | 1 | 2 | 3 | 4 | 5 | ? |
| My child is satisfied with his/her looks | Not at all 1 | 2 | Somewhat 3 | 4 | Very much 5 | Don't Understand 1 |

SPEECH AND LANGUAGE**In the past week, have:**

Other people had a hard time understanding your child when s/he talks?

Your child had a hard time understanding what other people say to him/her?

| | <i>Always</i> | <i>2</i> | <i>3</i> | <i>4</i> | <i>5</i> | <i>Never</i> | <i>Don't Understand</i> |
|--|---------------|----------|----------|----------|----------|--------------|-------------------------|
| Other people had a hard time understanding your child when s/he talks? | 1 | 2 | 3 | 4 | 5 | | ? |
| Your child had a hard time understanding what other people say to him/her? | 1 | 2 | 3 | 4 | 5 | | ? |

GLOBAL RATINGS

How well did the questions in this survey describe your child's quality of life?

How would you rate your child's overall quality of life?

How difficult was it to complete this survey?

How upsetting was it to complete this survey?

| | <i>Poor</i> | <i>2</i> | <i>3</i> | <i>4</i> | <i>5</i> | <i>Excellent</i> | <i>Don't Understand</i> |
|--|-------------|----------|----------|----------|----------|-------------------|-------------------------|
| How well did the questions in this survey describe your child's quality of life? | 1 | 2 | 3 | 4 | 5 | | ? |
| How would you rate your child's overall quality of life? | 1 | 2 | 3 | 4 | 5 | | ? |
| | <i>Very</i> | <i>2</i> | <i>3</i> | <i>4</i> | <i>5</i> | <i>Not at all</i> | <i>Don't Understand</i> |
| How difficult was it to complete this survey? | 1 | 2 | 3 | 4 | 5 | | ? |
| How upsetting was it to complete this survey? | 1 | 2 | 3 | 4 | 5 | | ? |

Thank You!

Please Put Your Completed Survey in the Stamped, Addressed Envelope and Drop in the Mail!

NF-1 Health-Related Quality of Life

Version 1.0

CHILD REPORT

DIRECTIONS:

Children with NF-1 sometimes have special problems. We are interested in how much of a problem these might have been for you in the PAST WEEK. There are no right or wrong answers! Just circle the number that answers the question. If the question doesn't make sense to you, just circle "I don't understand." Thanks!

GENERAL HEALTH**In general, I would say that my health is:**

| | | | | | | | |
|---|---|---|---|---|-----------|-----------|------------------|
| 1 | 2 | 3 | 4 | 5 | 1 Poor | Excellent | Don't Understand |
|---|---|---|---|---|-----------|-----------|------------------|

Compared to this time last year, I would say my health is:

| | | | | | | | |
|---|---|---|---|---|-----------------|-------------|------------------|
| 1 | 2 | 3 | 4 | 5 | 1 Much worse | Much better | Don't Understand |
|---|---|---|---|---|-----------------|-------------|------------------|

I get sick more easily than other kids:

| | | | | | | | |
|---|---|---|---|---|----------------------|----------------|------------------|
| 1 | 2 | 3 | 4 | 5 | 1 Yes, definitely | No, not at all | Don't Understand |
|---|---|---|---|---|----------------------|----------------|------------------|

APPETITE AND SLEEP**In the past week, have you:**

Had difficulty falling asleep or staying asleep?

| | | | | | | |
|---|---|---|---|---|-------|------------------|
| 1 | 2 | 3 | 4 | 5 | Never | Don't Understand |
|---|---|---|---|---|-------|------------------|

Had bad dreams or nightmares?

| | | | | | |
|---|---|---|---|---|---|
| 1 | 2 | 3 | 4 | 5 | ? |
|---|---|---|---|---|---|

Been less hungry than usual?

| | | | | | |
|---|---|---|---|---|---|
| 1 | 2 | 3 | 4 | 5 | ? |
|---|---|---|---|---|---|

Been hungrier than usual?

| | | | | | |
|---|---|---|---|---|---|
| 1 | 2 | 3 | 4 | 5 | ? |
|---|---|---|---|---|---|

UNDERSTANDING**In the past week, were you:****Bothered that you didn't know enough about NF-1?**

| | | | | | |
|------------|---|-----------|---|-----------------|-----------------------|
| 1 A lot | 2 | 3 Some | 4 | 5 Not at all | 1 Don't Understand |
|------------|---|-----------|---|-----------------|-----------------------|

Frustrated about having NF-1?

| | | | | | |
|------------|---|-----------|---|-----------------|-----------------------|
| 1 A lot | 2 | 3 Some | 4 | 5 Not at all | 1 Don't Understand |
|------------|---|-----------|---|-----------------|-----------------------|

SENSATION**In the past week, have you:**

Had difficulty hearing others when they talk?

Check here if you wear a hearing aid ____

Had trouble seeing well?

Check here if you wear glasses or contacts ____

Had trouble feeling the things you touch?

| | Always | 2 | Sometimes | 4 | 5 | Never | Don't Understand |
|---|--------|---|-----------|---|---|-------|------------------|
| 1 | 1 | 2 | 3 | 4 | 5 | ? | ? |
| 1 | 1 | 2 | 3 | 4 | 5 | ? | ? |
| 1 | 1 | 2 | 3 | 4 | 5 | ? | ? |

PAIN

In the past week, have you:

| | Always 1 | 2 | Sometimes 3 | 4 | Never 5 | Don't Understand ? |
|---|----------------|---|---------------------|---|-----------------|-----------------------|
| Hurt a lot? | | | | | | |
| Had muscle pains or aches | 1 | 2 | 3 | 4 | 5 | ? |
| Had headaches? | 1 | 2 | 3 | 4 | 5 | ? |
| Had stomachaches? | 1 | 2 | 3 | 4 | 5 | ? |
| Had pain in your bones or joints? | 1 | 2 | 3 | 4 | 5 | ? |
| Had neurofibromas or other areas on your body that hurt to touch? | 1 | 2 | 3 | 4 | 5 | ? |
| Compared to other people my age, the amount of pain I have is | Much more 1 | 2 | About the same 3 | 4 | Much less 5 | Don't Understand ? |
| Pain keeps me from doing the things I want to do or need to do | Very true 1 | 2 | Somewhat 3 | 4 | Not at all 5 | Don't Understand ? |

SYMPTOMS

In the past week, have you:

| | Always | 2 | Sometimes | 4 | 5 | Never | Don't Understand |
|---|--------|---|-----------|---|---|-------|------------------|
| Felt sick to your stomach? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had a headache? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had a stomachache? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had a hard time breathing? | 1 | 2 | 3 | 4 | 5 | | ? |
| Felt dizzy or like you might faint? | 1 | 2 | 3 | 4 | 5 | | ? |
| Felt weak? | 1 | 2 | 3 | 4 | 5 | | ? |
| Felt tired or fatigued? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had a hard time swallowing? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had a hard time keeping your balance? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had difficulty tying shoes, using scissors, holding a pencil? | 1 | 2 | 3 | 4 | 5 | | ? |
| Been clumsy? | 1 | 2 | 3 | 4 | 5 | | ? |
| Had a hard time riding a bike, running, or catching a ball? | 1 | 2 | 3 | 4 | 5 | | ? |

PSYCHOLOGICAL AND BEHAVIORAL-1

In the past week, have you:

| | Always | 2 | Sometimes | 3 | 4 | 5 | Never | Don't Understand | ? |
|---|--------|---|-----------|---|---|---|-------|------------------|---|
| Felt cranky or irritable? | 1 | 2 | 3 | 4 | 5 | | | | |
| Worried? | 1 | 2 | 3 | 4 | 5 | | | | |
| Felt anxious? | 1 | 2 | 3 | 4 | 5 | | | | |
| Gotten easily frustrated? | 1 | 2 | 3 | 4 | 5 | | | | |
| Gotten in trouble because of your behavior at school? | 1 | 2 | 3 | 4 | 5 | | | | |
| Gotten in trouble because of your behavior at home? | 1 | 2 | 3 | 4 | 5 | | | | |
| Felt afraid or scared? | 1 | 2 | 3 | 4 | 5 | | | | |
| Felt sad, down, or depressed? | 1 | 2 | 3 | 4 | 5 | | | | |
| Felt angry? | 1 | 2 | 3 | 4 | 5 | | | | |
| Worried about what might happen to you? | 1 | 2 | 3 | 4 | 5 | | | | |
| Felt like crying? | 1 | 2 | 3 | 4 | 5 | | | | |
| Felt lonely? | 1 | 2 | 3 | 4 | 5 | | | | |
| Felt cheerful? | 1 | 2 | 3 | 4 | 5 | | | | |

PSYCHOLOGICAL AND BEHAVIORAL -2

In the past week, have you:

| | Always | 2 | Sometimes | 4 | Never | 5 | Don't Understand |
|--|-----------------|---|-----------|---|------------------|---|------------------|
| Felt confident about yourself? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Enjoyed the things you do? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Had fun? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Felt jittery or restless? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Argued? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Wanted to be alone? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Had mood swings? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Not done what your parent or teacher asked? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Had anxiety or panic attacks? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Hit or kicked someone? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Heard voices that weren't there? | 1 | 2 | 3 | 4 | 5 | ? | ? |
| Compared to other children your age, would you say your behavior is | Much worse 1 | 2 | OK 3 | 4 | Much better 5 | 1 | Don't Understand |

SCHOOL AND COGNITION-1

In the past week, have you:

| | <i>Always</i> | <i>2</i> | <i>3</i> | <i>Sometimes</i> | <i>4</i> | <i>5</i> | <i>Never</i> | <i>Don't Understand</i> | <i>?</i> |
|--|---------------|----------|----------|------------------|----------|----------|--------------|-------------------------|----------|
| Missed school because of your health? Check here if school was on vacation _____ | 1 | 2 | 3 | 4 | 5 | | | | |
| Been bothered because you missed school? Check here if you haven't missed any school _____ | 1 | 2 | 3 | 4 | 5 | | | | |
| Missed school to go to the doctor or hospital? | 1 | 2 | 3 | 4 | 5 | | | | |
| Had difficulty solving math problems? | 1 | 2 | 3 | 4 | 5 | | | | |
| Had trouble writing papers or reports? | 1 | 2 | 3 | 4 | 5 | | | | |
| Had trouble following or understanding directions? | 1 | 2 | 3 | 4 | 5 | | | | |
| Had difficulty remembering what you read? | 1 | 2 | 3 | 4 | 5 | | | | |
| Had trouble reading? | 1 | 2 | 3 | 4 | 5 | | | | |
| Forgotten things? | 1 | 2 | 3 | 4 | 5 | | | | |
| Had trouble keeping up with your schoolwork? | 1 | 2 | 3 | 4 | 5 | | | | |
| Had trouble turning your schoolwork in on time? | 1 | 2 | 3 | 4 | 5 | | | | |

SCHOOL AND COGNITION--2

In the past week, have you:

| | Always 1 | 2 | Sometimes 3 | 4 | Never 5 | Don't Understand ? |
|---|-------------|---|----------------|---|----------------|-----------------------|
| Had difficulty paying attention and concentrating in class? | 1 | 2 | 3 | 4 | 5 | ? |
| Had trouble writing neatly? | 1 | 2 | 3 | 4 | 5 | ? |
| Had a hard time sitting still in class? | 1 | 2 | 3 | 4 | 5 | ? |
| Had trouble organizing your work or things? | 1 | 2 | 3 | 4 | 5 | ? |
| Had trouble spelling? | 1 | 2 | 3 | 4 | 5 | ? |
| How would you rate your ability to do your schoolwork? | Poor 1 | 2 | OK 3 | 4 | Excellent 5 | Don't Understand ? |

SOCIAL RELATIONS

In the past week, have you:

| | Always | 2 | Sometimes | 4 | Never | Don't Understand |
|--|--------|----|-----------|---|-------|------------------|
| Had trouble getting along with other kids? | 1 | 2 | 3 | 4 | 5 | ? |
| Helped others? | 1 | 2 | 3 | 4 | 5 | ? |
| Preferred to be alone? | 1 | 2 | 3 | 4 | 5 | ? |
| Preferred quiet activities? | 1 | 2 | 3 | 4 | 5 | ? |
| Avoided doing things with other kids? | 1 | 2 | 3 | 4 | 5 | ? |
| Felt bothered because you couldn't do the activities you like? | 1 | 2 | 3 | 4 | 5 | ? |
| Felt like other kids didn't want to do things with you? | 1 | 2 | 3 | 4 | 5 | ? |
| Felt like other kids didn't want to be your friend? | | | | | | |
| How would you rate your family's ability to get along with each other? | Poor | OK | 4 | 5 | 1 | Don't Understand |
| How would you rate your ability to get along with other kids? | 1 | 2 | 3 | 4 | 5 | 1 |

APPEARANCE

In the past week, have you:

| | Always | 2 | Sometimes | 4 | Never | Don't Understand |
|--|-----------------|---|---------------|---|----------------|-----------------------|
| Been teased about how you look? | 1 | 2 | 3 | 4 | 5 | ? |
| Felt bad about your appearance? | 1 | 2 | 3 | 4 | 5 | ? |
| Worried about how you look? | 1 | 2 | 3 | 4 | 5 | ? |
| Been teased about your size? | 1 | 2 | 3 | 4 | 5 | ? |
| Avoided doing things with others because of how you look? | 1 | 2 | 3 | 4 | 5 | ? |
| Done things to hide part of your body because of how it looks? | 1 | 2 | 3 | 4 | 5 | ? |
| Noticed people staring at you? | 1 | 2 | 3 | 4 | 5 | ? |
| Heard strangers make rude comments about you? | 1 | 2 | 3 | 4 | 5 | ? |
| Worried about other people teasing you? | 1 | 2 | 3 | 4 | 5 | ? |
| I am satisfied with the way I look | Not at all 1 | 2 | Somewhat 3 | 4 | Very much 5 | Don't Understand / |

APPETITE AND SLEEP

In the past week, have you:

| | <i>Always</i> | <i>2</i> | <i>3</i> | <i>Sometimes</i> | <i>4</i> | <i>5</i> | <i>Never</i> | <i>5</i> | <i>Don't Understand</i> | <i>?</i> |
|--|---------------|----------|----------|------------------|----------|----------|--------------|----------|-------------------------|----------|
| Had difficulty falling asleep or staying asleep? | 1 | 2 | 3 | 4 | 5 | 5 | 5 | 5 | 5 | ? |
| Had bad dreams or nightmares? | 1 | 2 | 3 | 4 | 5 | 5 | 5 | 5 | 5 | ? |
| Been less hungry than usual? | 1 | 2 | 3 | 4 | 5 | 5 | 5 | 5 | 5 | ? |
| Been hungrier than usual? | 1 | 2 | 3 | 4 | 5 | 5 | 5 | 5 | 5 | ? |

SPEECH AND LANGUAGE

In the past week, have:

Other people had a hard time understanding you when you talk?

You had a hard time understanding what other people say to you?

| | Always | 2 | 3 | Sometimes | 4 | 5 | Never | Don't Understand | ? |
|---|--------|---|---|-----------|---|---|-------|------------------|---|
| Other people had a hard time understanding you when you talk? | 1 | 2 | 3 | 4 | 5 | 5 | 5 | 5 | ? |
| You had a hard time understanding what other people say to you? | 1 | 2 | 3 | 4 | 5 | 5 | 5 | 5 | ? |

GLOBAL RATINGS

How well did the questions in this survey describe your quality of life?

| | Poor | 2 | 3 | Somewhat | 4 | 5 | Excellent | Don't Understand | ? |
|--|------|---|---|----------|---|---|-----------|------------------|---|
| How well did the questions in this survey describe your quality of life? | 1 | 2 | 3 | 4 | 5 | 5 | 5 | 5 | ? |

How would you rate your overall quality of life?

| | Poor | 2 | 3 | OK | 4 | 5 | Excellent | Don't Understand | ? |
|--|------|---|---|----|---|---|-----------|------------------|---|
| How would you rate your overall quality of life? | 1 | 2 | 3 | 4 | 5 | 5 | 5 | 5 | ? |

How difficult was it to complete this survey?

| | Very | 2 | 3 | Somewhat | 4 | 5 | Not at all | Don't Understand | ? |
|---|------|---|---|----------|---|---|------------|------------------|---|
| How difficult was it to complete this survey? | 1 | 2 | 3 | 4 | 5 | 5 | 5 | 5 | ? |

How upsetting was it to complete this survey?

| | Very | 2 | 3 | Somewhat | 4 | 5 | Not at all | Don't Understand | ? |
|---|------|---|---|----------|---|---|------------|------------------|---|
| How upsetting was it to complete this survey? | 1 | 2 | 3 | 4 | 5 | 5 | 5 | 5 | ? |

Thank You!

Please Put Your Completed Survey in the Stamped, Addressed Envelope and Drop in the Mail!